



3-28-07

Attorney Docket No. 07680.0018-00000

Jfw

## IN THE UNITED STATES PATENT AND TRADEMARK OFFICE

In re Application of:

Seng H. CHENG et al. )  
Application No.: 10/758,773 )  
Filed: January 16, 2004 ) Examiner: Shin-Lin CHEN  
For: COMBINATION ENZYME )  
REPLACEMENT, GENE ) Confirmation No.: 6298  
THERAPY AND SMALL )  
MOLECULE THERAPY FOR )  
LYSOSOMAL STORAGE )  
DISEASES )

CERTIFICATE UNDER 37 CFR § 1.10 OF  
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March 27, 2007

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By: Heather J. Morgan  
Heather J. Morgan

Commissioner for Patents  
P.O. Box 1450  
Alexandria, VA 22313-1450

Sir:

COPIES OF PREVIOUSLY CITED DOCUMENTS

Applicants enclose copies of 35 documents previously cited in an Information Disclosure Statement (IDS) filed on June 21, 2004 in the above-referenced application, but inadvertently omitted. Copies of the IDS filed on June 21, 2004 and of the corresponding Form PTO 1449 are also enclosed. Those 35 documents are listed below:

1. WO 00/62780

2. Abe et al., "Reduction of globotriaosylceramide in Fabry disease mice by substrate deprivation," *J. Clin. Invest.* 105:563, 2000.
3. Cox et al., "Novel oral treatment of Gaucher's disease with N-butyldeoxynojirimycin (06T 918) to decease substrate biosynthesis," *Lancet* 355:1481, 2000.
4. Deonarain et al., "Ligand-targeted receptor-mediated vectors for gene delivery," *Exp. Opin. Ther. Patents*, 8(1):53, 1998.
5. Desnick et al., "Fabry disease, an under-recognized multisystemic disorder: expert recommendations for diagnosis, management, and enzyme replacement therapy," *Ann. Intern. Med.* 138:338, 2003.
6. Eck et al., "Gene-based Therapy," in *Goodman and Gilman's The Pharmacological Basis of Therapeutics*, Ninth Edition, McGraw-Hill, New York, 1996, pp. 77-101.
7. Eng et al., "Safety and efficacy of recombinant human  $\alpha$ -galactosidase A replacement therapy in Fabry's disease," *N. Engl. J. Med.* 345:9, 2001.
8. Gorecki, "Prospects and problems of gene therapy: an update," *Expert Opin. Emerging Drugs*, 6(2):187, 2001.
9. Ioannou et al., "Fabry disease: preclinical studies demonstrate the effectiveness of  $\alpha$ -galactosidase A replacement in enzyme-deficient mice," *Am. J. Hum. Genet.* 68:14, 2001.
10. Jeyakumar et al., "Delayed symptom onset and increased life expectancy in Sandhoff disease mice treated with N-butyldeoxynojirimycin," *Proc. Natl. Acad. Sci. USA* 96:6388, 1999.

11. Jung et al., "Adeno-associated viral vector-mediated gene transfer results in long-term enzymatic and functional correction in multiple organs of Fabry mice," *Proc. Natl. Acad. Sci. USA* 98:2676.
12. Kakkis et al., "Overexpression of the human lysosomal enzyme  $\alpha$ -L-iduronidase in Chinese hamster ovary cells," *Protein Express. Purif.* 5:225, 1994.
13. Kakkis et al., "Long-term and high-dose trials of enzyme replacement therapy in the canine model of mucopolysaccharidosis I," *Biochem. Molec. Med.* 58:156, 1996.
14. Keeling et al., "Gentamicin-mediated suppression of Hurler syndrome stop mutations restores a low level of  $\alpha$ -L-iduronidase activity and reduces lysosomal glycosaminoglycan accumulation," *Hum. Mol. Genet.* 10:291, 2001.
15. Kikuchi et al., "Clinical and metabolic correction of Pompe disease by enzyme therapy in acid maltase-deficient quail," *J. Clin. Invest.* 101:827, 1998.
16. Lee et al., "Improved inhibitors of glucosylceramide synthase," *J. Biol. Chem.* 274:14662, 1999.
17. Medin et al., "Correction in trans for Fabry disease: expression, secretion and uptake of  $\alpha$ -galactosidase A in patient-derived cells driven by a high-titer recombinant retroviral vector," *Proc. Natl. Acad. Sci. USA* 93:7917, 1996.
18. Neufeld et al., "Lysosomal storage diseases," *Annu. Rev. Biochem.* 60:257, 1991.
19. Oshima et al., "Cloning, sequencing, and expressin of cDNA for human  $\beta$ -glucuronidase," *Proc. Natl. Acad. Sci. USA* 81:685, 1987.

20. Park et al., "Long-term correction of globotriaosylceramide storage in Fabry mice by recombinant adeno-associated virus-mediated gene transfer," *Proc. Natl. Acad. Sci. USA* 100:3450, 2003.
21. Pauly et al., "Complete correction of acid  $\alpha$ -glucosidase deficiency in Pompe disease fibroblasts in vitro, and lysosomally targeted expression in neonatal rat cardiac and skeletal muscle," *Gene Therapy* 5:473, 1998.
22. Platt et al., "Prevention of lysosomal storage in Tay-Sachs mice treated with *N*-butyldeoxynojirimycin," *Science* 276:428, 1997.
23. Ponce et al., "Enzyme therapy in Gaucher disease type 1: effect of neutralizing antibodies to acid  $\beta$ -glucosidase," *Blood* 90:43, 1997.
24. Schiffmann et al., "Enzyme replacement therapy in Fabry disease: a randomized controlled trial," *JAMA* 285:2743, 2001.
25. Shayman et al., "Inhibitors of glucosylceramide synthase," *Methods Enzymol.* 311:373, 2000.
26. Shull et al., "Enzyme replacement in a canine model of Hurler syndrome," *Proc. Natl. Acad. Sci. USA* 91:12937, 1994.
27. Takahashi et al., "E1B-55K-deleted adenovirus expressing E1A-13S by AFP-enhancer/promoter is capable of highly specific replication in AFP-producing hepatocellular carcinoma and eradication of established tumor," *Mol. Ther.* 5:627, 2002.
28. van der Ploeg et al., "Breakdown of lysosomal glycogen in cultured fibroblasts from glycogenosis type II patients after uptake of acid  $\alpha$ -glucosidase," *J. Neurol. Sci.* 79:327, 1987.

29. van der Ploeg, et al., "Intravenous administration of phosphorylated acid  $\alpha$ -glucosidase leads to uptake of enzyme in heart and skeletal muscle of mice," *J. Clin. Invest.* 87:513, 1991.
30. van der Ploeg et al., "Prospect for enzyme therapy in glycogenosis II variants: a study on cultured muscle cells," *J. Neurol.* 235:392, 1988.
31. van der Ploeg et al., "Receptor-mediated uptake of acid  $\alpha$ -glucosidase corrects lysosomal glycogen storage in cultured skeletal muscle," *Pediatr. Res.* 24:90, 1988.
32. Van Hove et al., "High-level production of recombinant human lysosomal acid  $\alpha$ -glucosidase in Chinese hamster ovary cells which targets to heart muscle and corrects glycogen accumulation in fibroblasts from patients with Pompe disease," *Proc. Natl. Acad. Sci. USA* 93:65, 1996.
33. Zaretsky et al., "Retroviral transfer of acid  $\alpha$ -glucosidase cDNA to enzyme-deficient myoblasts results in phenotypic spread of the genotypic correction by both secretion and fusion," *Hum. Gene Ther.* 8:1555, 1997.
34. Ziegler et al., "Correction of the nonlinear dose response improves the viability of adenoviral vectors for gene therapy of Fabry disease," *Hum. Gene Ther.* 13:935, 2002.
35. Ziegler et al., "Correction of enzymatic and lysosomal storage defects in Fabry mice by adenovirus-mediated gene transfer," *Hum. Gene Ther.* 10:1667, 1999.

Applicants respectfully request that the Examiner consider the listed documents and indicate that they were considered by making appropriate notations on PTO Form PTO 1449 submitted on June 21, 2004.

Application No. 10/758,773  
Attorney Docket No. 07680.0018-00000

If there are any fees due in connection with the filing of this paper, please charge the fees to Deposit Account No. 06-0916.

Respectfully submitted,

FINNEGAN, HENDERSON, FARABOW,  
GARRETT & DUNNER, L.L.P.

Dated: March 27, 2007

By:   
Konstantin M. Linnik  
Reg. No. 56,309  
Tel. (617) 452-1626



PATENT  
Customer No. 22,852  
Attorney Docket No. 07680.0018-00000

IN THE UNITED STATES PATENT AND TRADEMARK OFFICE

In re Application of: )  
Seng H. Cheng *et al.* ) Group Art Unit: 1632  
Application No.: 10/758,773 ) Examiner: Not yet assigned  
Filed: January 16, 2004 )  
For: Combination Enzyme ) Confirmation No.: 6298  
Replacement, Gene Therapy and )  
Small Molecule Therapy For )  
Lysosomal Storage Diseases )

**Mail Stop Amendment**  
Commissioner for Patents  
P.O. Box 1450  
Alexandria, VA 22313-1450

Sir:

**INFORMATION DISCLOSURE STATEMENT UNDER 37 C.F.R. § 1.97(b)**

Pursuant to 37 C.F.R. §§ 1.56 and 1.97(b), Applicants bring to the attention of the Examiner the documents listed on the attached PTO 1449. This Information Disclosure Statement is being filed before the mailing date of a first Office Action on the merits for the above-referenced application.

Copies of all listed documents, except U.S. patents, were submitted in prior U.S. Application No. 09/884,526.

This submission does not represent that a search has been made or that no better art exists and does not constitute an admission that each or all of the listed documents are material or constitute "prior art." If the Examiner applies any of the documents as prior art against any claim in the application and Applicants determine

PATENT  
Customer No. 22,852  
Attorney Docket No. 08702.0019-00000

that the cited documents do not constitute "prior art" under United States law, Applicant reserves the right to present to the Office the relevant facts and law regarding the appropriate status of such documents.

Applicants further reserve the right to take appropriate action to establish the patentability of the disclosed invention over the listed documents, should one or more of the documents be applied against the claims of the present application.

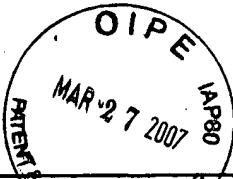
If there is any fee due in connection with the filing of this Statement, please charge the fee to our Deposit Account No. 06-0916.

Respectfully submitted,

FINNEGAN, HENDERSON, FARABOW,  
GARRETT & DUNNER, L.L.P.

Dated: 6/21/04

By: E Stewart Mittler  
E. Stewart Mittler  
Reg. No. 50,316



## INFORMATION DISCLOSURE CITATION

|  |                       |
|--|-----------------------|
| Atty. Docket No. 7680.0018-00000         | Appln. No. 10/758,773 |
| Applicant Seng H. Cheng and David Meeker |                       |
| Filing Date January 16, 2004             | Group: 1632           |

## U.S. PATENT DOCUMENTS

| Examiner Initial*     | Document Number | Issue Date | Name           | Class | Sub Class | Filing Date If Appropriate |
|-----------------------|-----------------|------------|----------------|-------|-----------|----------------------------|
| O L P E               | 6,066,626       | 05/23/00   | Yew et al.     | 514   | 44        |                            |
| E                     | 5,916,911       | 06/29/99   | Shayman et al. | 514   | 428       |                            |
| JUN 25 2004 C I N C Y | 5,945,442       | 08/31/99   | Shayman et al. | 514   | 428       |                            |
| C I N C Y             | 5,952,370       | 09/14/99   | Shayman et al. | 514   | 428       |                            |
| M A R K O F F I C E   | 6,030,995       | 02/29/00   | Shayman et al. | 514   | 428       |                            |
|                       | 6,040,332       | 03/21/00   | Shayman et al. | 514   | 428       |                            |
|                       | 6,051,598       | 04/18/00   | Shayman et al. | 514   | 428       |                            |
|                       | 5,840,702       | 11/24/98   | Bedwell        | 514   | 23        |                            |
|                       | 6,696,059       | 06/24/04   | Jacob et al.   | 424   | 94.61     |                            |
|                       | 6,495,570       | 12/17/02   | Jacob et al.   | 514   | 328       |                            |
|                       | 20030017139     | 01/23/03   | Souza et al.   | 424   | 93.3      | 05/06/02                   |
|                       | 20020142985     |            | Dwek et al.    | 514   | 44        | 10/19/01                   |
|                       | 20010044453     |            | Jacob et al.   | 514   | 320       | 05/17/01                   |
|                       | 20020127213     |            | Jacob et al.   | 424   | 94.1      | 01/22/02                   |

## FOREIGN PATENT DOCUMENTS

|  | Document Number | Publication Date | Country | Class | Sub Class | Translation Yes or No |
|--|-----------------|------------------|---------|-------|-----------|-----------------------|
|  | WO 00/09153 *   | 02/24/00         | PCT     |       |           |                       |
|  | WO 00/62779 *   | 10/26/00         | PCT     |       |           |                       |
|  | WO 00/62780     | 10/26/00         | PCT     |       |           |                       |

## OTHER DOCUMENTS (Including Author, Title, Date, Pertinent Pages, Etc.)

|  |   |
|--|---|
|  | Abe et al., "Reduction of globotriaosylceramide in Fabry disease mice by substrate deprivation," J. Clin. Invest. 105:563, 2000.                                |
|  | Abe et al., "Glycosphingolipid depletion in Fabry disease lymphoblasts with potent inhibitors of glucosylceramide synthase," Kidney International 57:446, 2000. |
|  | Cox et al., "Novel oral treatment of Gaucher disease with N-leutyldeoxyinojirimycin (06T 918) to decease substrate biosynthesis," Lancet 355:1481, 2000.        |
|  | Deonarain et al., Exp. Opin. Ther. Patents, 8(1):53,1998.   |

## INFORMATION DISCLOSURE CITATION

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| Atty. Docket No. | 07680.0018-00000               | Appln. No. | 10/758,773 |
| Applicant        | Seng H. Cheng and David Meeker |            |            |
| Filing Date      | January 16, 2004               | Group:     | 1632       |

## OTHER DOCUMENTS (Including Author, Title, Date, Pertinent Pages, Etc.)

|  |   |
|--|---|
|  | Desnick et al., "Fabry disease, an under-recognized multisystemic disorder: expert recommendations for diagnosis, management, and enzyme replacement therapy," Annals Int. Med. 138:338, 2003.  |
|  | Eck et al., Goodman and Gilman's The Pharmacological Basis of Therapeutics, Ninth Edition, McGraw-Hill, New York, 1996, p 77-101.   |
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|  | Kakkis et al., "Overexpression of the human lysosomal enzyme alpha-L-iduronidase in Chinese hamster ovary cells," Prot. Express. Purif. 5:225, 1994.  |
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|  | Kikuchi et al., "Clinical and metabolic correction of pompe disease by enzyme therapy in acid maltase-deficient quail," J. Clin. Invest. 101:827, 1998.   |
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|  | Oshima et al., "Cloning, sequencing, and expressin of cDNA for human beta-glucuronidase," Proc. Natl. Acad. Sci. USA 81:685, 1987.  |
|  | Overkleef et al., "Generation of specific deoxynojirimycin-type inhibitors of the non-lysosomal glucosylceramidase," J. Biol. Chem. 273(41):26522,1998.   |
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|  | Schiffmann et al., "Infusion of alpha-galactosidase A reduces tissue globotriacylglycerol storage in patients with Fabry disease," <i>Proc. Natl. Acad. Sci. USA</i> , 97(1):365, 2000.  |
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|  | Shull et al., "Enzyme replacement in a canine model of Hurler syndrome," <i>Proc. Natl. Acad. Sci. USA</i> 91:12937, 1998.   |
|  | Takahashi et al., "E1B-55K-deleted adenovirus expressing E1A-13S by AFP-enhancer/promoter is capable of highly specific replication in AFP-producing hepatocellular carcinoma and eradication of established tumor," <i>Mol. Ther.</i> 5:627, 2002.  |
|  | Van Der Ploeg et al., "Breakdown of lysosomal glycogen in cultured fibroblasts from glycogenosis type II patients after uptake of acid alpha-glucosidase," <i>J. Neurol. Sci.</i> 79:327, 1987.  |
|  | Van Der Ploeg, et al., "Intravenous administration of phosphorylated acid alpha-glucosidase leads to uptake of enzyme in heart and skeletal muscle of mice," <i>J. Clin. Invest.</i> 87:513, 1991.   |
|  | Van Der Ploeg et al., "Prospect for enzyme therapy in glycogenosis II variants: a study on cultured muscle cells," <i>J. Neurol.</i> 235:392, 1998.  |
|  | Van Der Ploeg et al., "Receptor-mediated uptake of acid alpha-glucosidase corrects lysosomal glycogen storage in cultured skeletal muscle," <i>Pediatr. Res.</i> 24:90, 1988.  |
|  | Van Hove et al., "High-level production of recombinant human lysosomal acid alpha-glucosidase in Chinese hamster ovary cells which targets to heart muscle and corrects glycogen accumulation in fibroblasts from patients with Pompe disease," <i>Proc. Natl. Acad. Sci. USA</i> 93:65, 1996. |
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| Filing Date      | January 16, 2004   | Group:     | 1632       |
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